Early initiation of eculizumab treatment in patients with atypical haemolytic uraemic syndrome improves long-term outcomes: pooled analysis of clinical trials

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Introduction

Atypical haemolytic uraemic syndrome (aHUS) is a severe, life-threatening disease requiring rapid treatment to inhibit complement-mediated thrombotic microangiopathy (TMA) and avoid irreversible organ damage. Four prospective clinical trials have reported the safety and efficacy of eculizumab (Ecu) in the treatment of aHUS [1,2]. We report data from a pooled analysis of these trials on renal function in patients (pts) starting Ecu within ≤7 days or >7 days after the current aHUS manifestation.

Methods

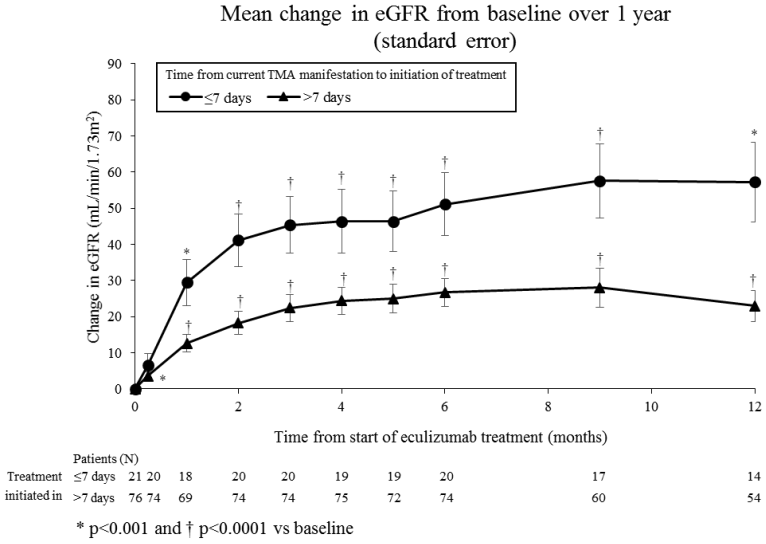
Data from four phase 2, open-label, single-arm trials including both paediatric and adult pts with aHUS were pooled. Pts with a documented date of onset of current TMA manifestation and a baseline estimated glomerular filtration rate (eGFR) of <90 mL/min/1.73m2 were included. Changes from baseline in eGFR were analysed at study visits using a one-sample t-test.

Results

Data from 97 pts were analysed: median (range) age at enrolment was 29 (0–80) years; 62% of pts were females; median (range) duration of current manifestation to start of Ecu treatment was 23 (1–1447) days; median (range) baseline eGFR was 15.9 (5.6–76.1) mL/min/1.73m^2. Ecu treatment was started in 21 pts in ≤7 days and 76 pts in >7 days after presentation with TMA. Median eGFR was 11 mL/min/1.73m^2 for the pts started within 7 days and 16 for those initiating >7 days. The mean change from baseline in eGFR for pts starting Ecu in ≤7 days and in >7 days after presentation with TMA are 57 and 23 mL/min/1.73m^2 at 1 year, respectively (Figure).

Conclusions

This pooled analysis indicates that pts treated with Ecu within 7 days of a TMA manifestation had a greater improvement in eGFR over time than pts in whom treatment was delayed. These data show the importance of rapid diagnosis and treatment of aHUS for recovery of renal function.



* p<0.001 and † p<0.0001 vs baseline

References

1. Legendre C, et al. NEJM 2013;368:2169-81
2. Keating GM. Drugs 2013;73:2053-66

Character count: Approx 2980

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